

CIRM Funded Clinical Trials

Efficacy and safety of cryopreserved autologous CD34+ HSC transduced with EFS lentiviral vector encoding for human ADA gene in ADA-SCID subjects

Disease Area:

Adenosine deaminase-deficient Severe Combined Immunodeficiency (ADA-SCID)

Investigator:

Donald Kohn

Institution:

University of California, Los Angeles

CIRM Grant:

CLIN2-09339

Award Value:

\$19,065,745

Trial Sponsor:

University of California, Los Angeles

Trial Stage:

Phase 2

Trial Status:

Recruiting

Targeted Enrollment:

10

ClinicalTrials.gov ID:

NCT02999984



Donald Kohn

Details:

In ADA-SCID, allogeneic hematopoietic (blood) stem cell transplants from non-matched sibling donors are a high risk procedure. Additionally, the efficacy of chronic enzyme replacement therapy is uncertain in the long-term. A team at UCLA is using a patient's own blood stem cells to try and rebuild the damaged immune systems of patients with ADA-SCID. They will use what's called a lentiviral vector to deliver genetic material into the blood stem cells, correcting the genetic flaw that causes SCID. It's hoped this will create a new blood system and a healthy immune system. Preliminary data indicates that OTL-101 - a stem cell gene therapy developed by UCLA and Orchard Therapeutics Limited - may significantly improve outcomes compared to available therapies.

Design:

Comparability of cryopreserved product versus fresh product.

Goal:

Primary: Safety. Secondary: Efficacy, gene marking, immune reconstitution. Registrational trial.

Updates:

Breakthrough Therapy Designation. Rare Pediatric Designation. Early evidence of safety and clinical efficacy in all treated patients.

News Releases:

Pioneering stem cell gene therapy cures infants with bubble baby disease

Study analyzes safety and effectiveness of stem cell gene therapy for bubble baby disease

Orchard Therapeutics announces that OTL-101 has received a Rare Paediatric Disease Designation

Source URL: <http://www.cirm.ca.gov/clinical-trial/efficacy-and-safety-cryopreserved-autologous-cd34-hsc-transduced-efs-lentiviral>